Abstract

A composition for delivery of Factor VIII is described. The composition is comprised of an expression vector encoding for Factor VIII and a lipid represented by the formula:

$$Z$$
 $\bigcap_{n} L$ $\bigcap_{0} R^{2}$ R^{1}

where each of R^1 and R^2 is an alkyl or alkenyl chain having between 8-24 carbon atoms; n = 0-20, preferably 1-20; L is selected from the group consisting of (i) -X-(C=O)-Y-, (ii) -X-(C=O)-, and (iii) -X-, where X and Y are independently selected from oxygen, NH and a direct bond; and Z is a weakly basic moiety that has a pK of less than 7.4 and greater than about 4.0.